NIH-RAID Program

(Rapid Access to Interventional Development)

PAR-09-027

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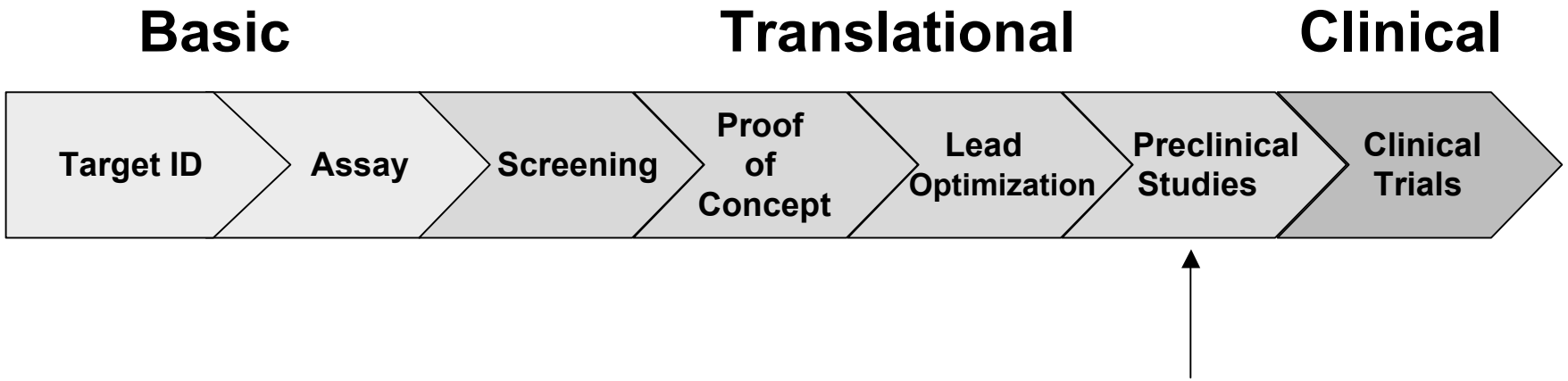
NIH-RAID Program

Position in Drug Development Pipeline



NCI-RAID/NExT
T1D-RAID
NIH-RAID

Access to contract services

- Synthesis
- Formulation
- In vivo PK and Tox studies
- Product development planning

NIH-RAID Program Overview

- The NIH Roadmap established NIH-RAID Program to make available, on a competitive basis, certain critical resources needed for the development of new therapeutic agents
- Applications are accepted for the development of therapies for all diseases
- Lead by OSC, NINDS & NIAMS, project team
- Began accepting applications in 2005

NIH-RAID Program Overview

- X01 Resource Access Award
- Approved projects are provided access to the expertise and contract resources of NCI and NHLBI programs
- Open to domestic and foreign academic and non-profit institutions, as well as SBIR-eligible businesses, and NIH Intramural programs
- Small molecules, peptides, oligonucleotides, natural products, gene vectors, monoclonal antibodies and recombinant proteins are eligible for development
- IP retained by owner
- R&D costs split 50/50 between Roadmap and ICs (IR dollars must be used for IR applicants)

NIH-RAID Services

- Synthesis in bulk of small molecules
- Synthesis of oligonucleotides
- Chemical synthesis of peptides
- Synthesis of gene vectors
- Scale-up production
- Development of analytical methods
- Isolation and purification of natural products
- Development of suitable formulations
- Manufacture of clinical trial drug supplies
- Pharmacokinetic/ADME studies including bioanalytical method development*
- Range-finding initial toxicology*
- IND-directed toxicology*
- Product development planning and advice in IND preparation

Entry Points

- Lead Molecule Identified
  - NIH-RAID X01 does not provide lead optimization or animal efficacy services
- Lead Molecule Not Identified
  - Scale-up Synthesis
  - Preliminary PK/Tox

Application & Approval Process

- Three receipts per year
- CSR review
- Preliminary cost estimate from NCI/NHLBI
- Investigator seminar, if warranted
- Preparation of tasks, timeline, milestones, and costs by NCI
- Funding decisions by co-sponsoring ICs
- Applications do not go to council
- Total time from submission to approval : 10+ months
- Independent Product Development Plans available

Project Management

- NIH-RAID Material Transfer Agreements
- IC transfers funds to the NCI or NHLBI
  - 50% RM and 50% IC funds
- Projects typically last two years
  - Sequential development
  - Spreads costs across fiscal years, out years committed
- Monthly progress meetings and reports
- ICs in control at decision points
  - Go/no go
  - Insufficient funds

Results to Date

- 105 total applications submitted since 2005
  - 17 approved
  - 29% approval rate
- Status of approved projects
  - 7 completed projects
  - 5 successful INDs (not all projects lead to IND)
  - 10 active, 2 additional INDs expected by Q1 2010
- 10 ICs have co-funded a project

Examples of Approved Projects

- Development of HGF Mimetic (Refanalin) for Hepatic Fibrosis
- Inhibitors of Glutaminase 2 as Therapeutic Agents for Neuro-Oncological Diseases and Celiac Sprue
- Safety Pharmacology Studies for an IND for Beta Thalassemia
- Metastin Administration in Humans: Preclinical Toxicology Studies
- Redox Encrypted Therapeutics for Treatment of Friedreich's Ataxia
- Advanced Studies with 5HMF –Potent Antisickling Agent
- Preclinical Development of CDD-0102 Treatment of Alzheimer's disease
- cGMP Synthesis of Selective Kappa Opioid Receptor Antagonist JDTic

Efficacy Administrative Supplements

- Jan/Feb 2010 receipt date
- Any research project (Rs,Ps,Us) with a year of funding remaining
- In vitro or in vivo efficacy
- $2M Budget
  - 100% Roadmap funds
  - 50K direct costs
  - up to 25 awards made

Future Funding

- X01 Applications accepted through Sept 2011
- Approved projects completed through 2013
- Efficacy Supplements accepted in FY10 and FY11

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